



Voretigene Neparvovec for Biallelic RPE65-Mediated Retinal Disease: Effectiveness and Value

Draft Voting Questions for January 25, 2018 Public Meeting

These questions are intended for the deliberation of the Midwest CEPAC voting body at the public meeting.

Comparative Clinical Effectiveness

- 1) Is the evidence adequate to demonstrate that the net health benefit of treatment with voretigene neparvovec is greater than that of supportive care?

Yes No

Other Benefits

- 2) When **compared to best supportive care**, does voretigene neparvovec offer one or more of the following “other benefits”? (yes, no, uncertain)
- This intervention provides significant direct patient health benefits that are not adequately captured by the QALY.
 - This intervention offers reduced complexity that will significantly improve patient outcomes.
 - This intervention will reduce important health disparities across racial, ethnic, gender, socioeconomic, or regional categories.
 - This intervention will significantly reduce caregiver or broader family burden.
 - This intervention offers a novel mechanism of action or approach that will allow successful treatment of many patients who have failed other available treatments.
 - This intervention will have a significant impact on improving patients’ ability to return to work and/or their overall productivity.
 - This intervention will have a significant positive impact outside the family, including on schools and/or communities.
 - This intervention will have a significant impact on the entire “infrastructure” of care, including effects on screening for affected patients, on the sensitization of clinicians, and on the dissemination of understanding about the condition, that may revolutionize how patients are cared for in many ways that extend beyond the treatment itself.
 - There are other important benefits or disadvantages that should have an important role in judgments of the value of this intervention: _____

Contextual Considerations

- 3) Are any of the following contextual considerations important in assessing voretigene neparvovec's long-term value for money in patients? (yes, no, uncertain)
- a. This intervention is intended for the care of individuals with a condition of particularly high severity in terms of impact on length of life and/or quality of life.
 - b. This intervention is intended for the care of individuals with a condition that represents a particularly high lifetime burden of illness.
 - c. This intervention is the first to offer any improvement for patients with this condition.
 - d. Compared to supportive care, there is significant uncertainty about the long-term risk of serious side effects of this intervention.
 - e. Compared to supportive care, there is significant uncertainty about the magnitude or durability of the long-term benefits of this intervention.
 - f. There are additional contextual considerations that should have an important role in judgments of the value of this intervention: _____.

Long-Term Value for Money

- 4) Given the available evidence on comparative clinical effectiveness and incremental cost effectiveness, and considering other benefits and contextual considerations, what is the long-term value for money of voretigene neparvovec compared with supportive care?

High

Intermediate

Low